Title (en)

AAV-MEDIATED GENE THERAPY FOR RPGR X-LINKED RETINAL DEGENERATION

Title (de)

AAV-VERMITTELTE GENTHERAPIE ZUR X-CHROMOSOMALEN NETZHAUTDEGENERATION

Title (fr)

THÉRAPIE GÉNIQUE INDUITE PAR VAA S'APPLIQUANT À LA DÉGÉNÉRESCENCE RÉTINIENNE LIÉE À L'X DE RPGR

Publication

EP 2872183 A4 20151209 (EN)

Application

EP 13816245 A 20130123

Priority

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- US 2013022628 W 20130123

Abstract (en)

[origin: WO2014011210A1] Described herein are methods of preventing, arresting progression of or ameliorating vision loss and other conditions associated with retinitis pigmentosa and x- linked retinitis pigmentosa in a subject. The methods include administering to said subject an effective concentration of a composition comprising a recombinant adeno -associated virus (AAV) carrying a nucleic acid sequence encoding a normal retinitis pigmentosa GTPase regulator (RPGR gene), or fragment thereof, under the control of regulatory sequences which express the product of the gene in the photoreceptor cells of the subject, and a pharmaceutically acceptable carrier.

IPC 8 full level

A61K 48/00 (2006.01); **A61K 38/17** (2006.01); **A61K 38/46** (2006.01); **A61P 27/02** (2006.01); **C07K 14/47** (2006.01)

CPC (source: EP US)

A61K 38/1709 (2013.01 - EP US); **A61K 38/185** (2013.01 - EP US); **A61K 38/46** (2013.01 - EP US); **A61K 48/005** (2013.01 - EP US); **A61P 27/02** (2018.01 - EP); **A61P 43/00** (2018.01 - EP); **C07K 14/4702** (2013.01 - EP US); **C12N 15/86** (2013.01 - US); A61K 48/00 (2013.01 - US); C12N 2750/14143 (2013.01 - EP US); C12N 2830/008 (2013.01 - EP US); C12Y 306/05 (2013.01 - EP US)

Citation (search report)

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- [XY] M. NATKUNARAJAH ET AL: "AAV Mediated Gene Replacement Therapy in the RPGR Knockout Mouse- A Model of X-Linked Retinitis Pigmentosa", INVESTIGATIVE OPHTHALMOLOGY & VISUAL SCIENCE - IOVS, vol. 46, no. Suppl. S, 1 May 2005 (2005-05-01), US, pages 5224, XP055223624, ISSN: 0146-0404
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- See also references of WO 2014011210A1

Designated contracting state (EPC)

AL AT BE BG CH CY CZ DE DK EE ES FI FR GB GR HR HU IE IS IT LI LT LU LV MC MK MT NL NO PL PT RO RS SE SI SK SM TR

DOCDB simple family (publication)

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DOCDB simple family (application)

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